DEPARTMENT OF HEALTH, EDUCATION, AND WELFARE

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In reply refer to:
NBI-D

Dr. Joshua Lederberg
Department of Genetics
Stanford University
Medical Center
Palo Alto, California

Dear Dr. Lederberg:

Your letter of March 6 raises some very interesting questions regarding the evaluation of drugs prior to their release for general use. As you may have noted, a tranquilizing agent in common use in Germany has recently been demonstrated to be responsible for an "epidemic" of an unusual form of congenital malformation.

It is quite evident that the use of anticholesterol agents has serious hazards. I understand that triparanol has been withdrawn from public use because of untoward side effects--particularly cataract.

One of the most serious concerns to which I have recently become sensitive has to do with the use of drugs during early pregnancy. It would seem that any proposed new therapeutic agent should be tested for its effect on the fetus before being released for general use.

Dr. Roscoe Brady of our Institute is particularly concerned with fatty acid synthesis in the brain and actually has a manuscript in preparation dealing with investigations on the inhibition of fatty acid synthesis by brain enzymes. This is not quite the identical problem to which you refer in your letter, but is in a closely related field.

The whole problem of evaluation of new drugs--not only for toxicity but also for effectiveness--is a source of serious concern to this Institute. There is a constant stream of proposed new therapies for

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mental deficiency. Some of these obviously are "quackery"--for others, there is a sufficient kernel of possible usefulness to cause us to feel some obligation to conduct a therapeutic trial. However, few scientists have the enthusiasm to involve themselves in the expenditure of time and energy which is required to conduct a good field trial of a proposed new therapy--certainly unless there is strong presumptive evidence that such a therapy should be useful. Meanwhile, the public is spending millions of dollars for useless treatments. It has thus developed that one of our major worries has to do with the evaluation of newly recommended therapies, and we are laying the groundwork for clinical trials in certain limited areas.

Thank you very much for bringing to my attention this particular specific problem which certainly relates very closely to our area of interest.

Sincerely yours,

Richard L. Masland, M. D.
Director, National Institute of
Neurological Diseases and Blindness